



# Pharmacoeconomics and Outcomes Research – What is it and how is it being applied in the South African health care system?

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The last three decades has seen the rapid development of new medical technologies in both the pharmacological and devices industries. New health care technology has come, in many instances, with a new price tag, which has left many national and private funding agencies struggling to decide how to use the funds available in the “best” way possible.

It has been out of this need for evaluation of current and new treatment options that the disciplines of pharmacoeconomics and outcomes research emerged. Health economics and the issues around health care financing have been topics of some debate for many years, and the term pharmacoeconomics was first coined in the 1960s, as a subdivision of health economic arguments.

Any form of economics and economic analysis stems from the single, and very real fact that resources are scarce and the demand inevitably exceeds the resources available. In health care this is no different – pressures on the resources available for the provision of health care are constantly increasing. Economics is predominantly concerned with achieving efficient use of the resources available. That is, the decision of how to make the best possible use of the resources available for competing options, to achieve the greatest outcomes for patients, at the least possible cost.

Outcomes research and pharmacoeconomics are put together as part of health technology assessment – a process which is gaining momentum both abroad and in South Africa, in the decision-making processes in the health care environment.

### Pharmacoeconomics

The International Society for Pharmacoeconomics and Outcomes Research (ISPOR), considers pharmacoeconomics to be a scientific discipline that evaluates the clinical, economic and humanistic aspects of pharmaceutical products, services, and programmes, as well as other health care interventions. The primary purpose of pharmacoeconomic research and analysis is to provide assistance to health care decision makers, providers and patients for optimal outcomes and the allocation of health care resources.<sup>1</sup>

By its nature, pharmacoeconomics has to be multi-disciplinary, incorporating such aspects as health economics, clinical evaluations, risk analysis, technology assessment, and health-related quality of life and epidemiology in the examination of

pharmacological agents, medical devices, diagnostics, surgery and disease-prevention services.

In recent years, the use and recognition of pharmacoeconomics as an academic discipline and a practically useful tool, has grown rapidly. Research in 2001 from the Tufts Centre for the Study of Drug Development suggested that the demand for pharmacoeconomic analyses conducted by the pharmaceutical industry was likely to grow substantially from the spending at the time (average 1% of pharmaceutical research and development cost) in the near future.<sup>2</sup>

This investment is potentially well justified – in countries where there is a national form of health insurance, or a national health system serving the public, economic evaluation often takes centre-stage along with other aspects of a new treatment. There are many countries worldwide, which have issued national guidelines for economic assessment for new technologies, and manufacturers who hope to have their new technology included into current funding structures have to robustly justify their place amongst other available treatments.

### Outcomes Research

One of the critical, and most fascinating components of economic evaluation in health care is that of outcomes research. Outcomes research is really the clinical aspect of any pharmacoeconomic evaluation.

ISPOR's definition of outcomes research is that it is a scientific discipline that evaluates the effect of health care interventions on patient health status, involving economic, clinical, or humanistic outcomes.<sup>1</sup>

The term “outcomes” essentially relates to consequences of an intervention and may relate to an array of data. Clinical/physiological, clinician-reported, care-giver reported or patient-reported outcomes can be utilised to capture aspects such as overall symptom improvement, patient condition, response to treatment, health related quality of life, satisfaction with treatment and patient adherence and compliance to therapy.<sup>1</sup>

The instruments for health outcomes research are powerful, but complex, and frequently, the outcomes from a particular intervention can only be evaluated over several years. This is one of the reasons why, in outcomes research, the distinction is made

tween outcomes, i.e. end results, and intermediate endpoints, e.g. surrogate endpoints. For example, in the treatment of hypertension, the measure of blood pressure is a *clinical intermediary*, while the *clinical outcomes* which we are ACTUALLY trying to prevent by giving treatment are, for example stroke or myocardial infarctions.

Sometimes, like in this hypertension example, the link between the intermediate endpoint and the ultimate clinical outcome is quite well established, but in other diseases, which are less well researched, this may not be the case.

Indeed, the perception of an outcome frequently depends on the perspective that is used. For example, if a pharmacist is implementing a counselling programme, hoping to assist patients to achieve better compliance with their antihypertensive drug treatment, compliance would be viewed as an "end result" from the pharmacist's perspective. However, from the perspective of a researcher studying stroke, compliance may be viewed as an intermediary while the ultimate outcome of interest is stroke reduction or survival.

The increasing interest in outcomes research has generated new guidelines overseas, the FDA having issued their final guidance on the use of patient reported outcomes in pharmacological research in December 2009.<sup>3</sup> The guidelines have added credibility to outcomes research as a discipline, and will inevitably see patient reported outcomes being included in clinical trials as part of measurement of efficacy and safety of pharmacological therapies.<sup>4</sup>

### Health Technology Assessment (HTA)

Health technology assessment (HTA) is a form of policy research that examines short- and long-term consequences of the application of a health care technology.

The goal of HTA is to provide policymakers with information on policy alternatives. For any given technology, properties and impacts assessed may include technical properties (this is particularly germane for sophisticated equipment), evidence of safety, efficacy (including patient-reported outcomes), real-world effectiveness, cost, and cost-effectiveness as well as estimated social, legal, ethical, and political impacts.

A number of countries have set up government agencies dedicated to HTA, and it is being utilised increasingly widely as an aid to decision-making. Up until now, much of the focus has been on pharmaceuticals, but the structures are fast adapting to be able to include devices, procedures and diagnostic techniques.

A key issue associated with HTA, at least as it has been practiced at the governmental level (which has been its main focus worldwide) has been the lack of collaboration between the innovators, producers and advocates of health technology on the one hand and the governmental policymakers, who are the consumers of HTA, on the other hand, and whose charge is to decide on societal adoption, use and reimbursement of health care technologies.

### Pharmacoeconomics, outcomes research and health technology assessment in South Africa

It is probably safe to say that pharmacoeconomics and outcomes research are still in their infancy in South Africa, but the interest from all stakeholders in the South African health care environment, both public and private sector, has picked up rapidly in the last decade.

Most pharmaceutical companies in SA now have units dedicated to what has been termed "market access", which involves all of the aspects of pharmacoeconomic evaluation, outcomes research and technology assessment.

Many of the private funders are performing HTA and pharmacoeconomic assessment on new technologies and drugs as they come to market to decide how to fund them for fund members, in the face of ever-increasing costs of private health care.

The South African Department of health is also increasingly using input from pharmacoeconomic studies and principles to assist them in making decisions as to what to include in standard treatment guidelines and on medication lists within the government-funded health care system.

These decisions impact pharmacists and their patients on a daily basis in terms of formulary listing for medications, reimbursement policies for novel treatments, devices and procedures, and it is becoming increasingly important and useful in daily practice, for health care providers to grasp at least the basics of the thinking behind the principles applied.

*The International Society for Pharmacoeconomics and Outcomes Research South Africa (ISPOR SA) was founded in 2007, with a view to developing the skills, recognition and utilisation of pharmacoeconomics, outcomes research and health technology assessment in South Africa. The society's mission is to improve the standard of pharmacoeconomic and outcomes research by promoting research, education, training and providing leadership towards optimal healthcare policies and standards. ISPOR SA currently has a membership of 85 individuals, from academia, pharmaceutical and devices industries, health care funders, and practising pharmacists and doctors. The Society holds an annual conference (in September in 2010), and there is continuous activity in the form of working groups addressing important topics in health economics, drug pricing and reimbursement issues, in the South African context. □*

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